Clinical Trials to Help Kidney Transplant Patients and Defeat Blindness Get Support from Stem Cell Agency Board

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Oakland, CA – A simple injection of stem cells that could help kidney transplant patients avoid a lifetime on anti-rejection drugs was one of two new clinical trials approved today by the governing Board of the California Institute for Regenerative Medicine (CIRM).

There are currently more than 100,000 people in the US waiting for a life-saving kidney transplant. Even if they get the organ they face a lifetime on powerful, immunosuppressive drugs, which prevent the body from rejecting the transplant, but which also increase the risk of cancer, infection and heart disease. Now researchers at Stanford University have been awarded $6.65 million to see if injecting blood stem cells and T cells (which plays an important role in the immune system) from the kidney donor into the kidney recipient can enable the recipient to bypass the need for these drugs.

“Every year around 17,000 kidney transplants take place in the US, but getting one of these life-saving organs doesn’t mean life is now going to be easy for the recipients,” says C. Randal Mills, Ph.D., President and CEO of CIRM. “Many transplant patients experience fevers, infections and other side effects as a result of taking the anti-rejection medications. This clinical trial is a potentially transformative approach that could help protect the integrity of the transplanted organ, and improve the quality of life for the kidney recipient.”

The CIRM Board also approved almost $8.3 million in funding for a clinical trial using stem cells to treat a rare form of blindness called retinitis pigmentosa (RP). This disease, which often strikes people before the age of 30, destroys the light sensing cells at the back of the eye. There is no cure.

jCyte Inc. is treating RP by using retinal progenitor cells, injected into the back of the eye. These cells secrete factors that will protect undamaged cells and may even repair those damaged by the disease. They have already shown this approach is safe and potentially beneficial in a small CIRM-funded Phase 1 clinical trial. Now they want to try the same approach with a larger group of patients.

These two projects bring the number of new clinical trials funded by CIRM this year to ten. That is the same number that CIRM-funded in the first ten years of its existence.

“Exactly one year ago our Board approved a new Strategic Plan, laying out some ambitious goals for the agency over the next five years,” says Mills, “One of those goals was to fund ten new clinical trials this year. We knew this would be a tough goal to achieve, but when patients’ lives are on the line our team steps up and delivers. We are proud of everyone who helped make this happen, from the Board and our great scientific reviewers to every member of the CIRM team.”

Four people who have been part of CIRM-supported research attended the meeting to thank the Board for the work that has helped change and, in two cases, helped save, their lives.

Four-year-old Evangelina Padilla-Vaccaro and 22-year-old Brenden Whittaker were both born with immune disorders that left them with limited ability to fight off infections. Both diseases are life-threatening and both were cured thanks to work by Dr. Don Kohn at UCLA, using a gene modified stem cell therapy.

Evangelina’s mother, Alysia, expressed her gratitude to the the CIRM Board, saying: “Thank you for keeping my family complete.”

Jake Javier and Karl Trede also talked about their experiences in CIRM-funded clinical trials. Jake had ten million stem cells transplanted into his neck after suffering a severe spinal cord injury this summer. Karl became the first patient treated in an anti-cancer trial out of Stanford University.

Jake said the transplant has given him hope he will regain the full use of his arms and hands, and be able to lead an independent life.

The Board also approved the re-appointments of Jonathan Thomas, Ph.D., J.D., Chair of the Board and Sen. Art Torres (Ret.) Vice Chair of the Board, and honored Dr. Michael Friedman who is stepping down after ten years on the CIRM Board. Dr. Friedman was praised for his

“experience, commitment, knowledge, and leadership,” saying this work “contributed greatly to the momentum of discovery and the future therapies which will be the ultimate outcome of the dedicated work of the researchers receiving CIRM funding.”

Doctor Friedman said he was deeply moved by the tributes, and treasured being part of CIRM: “I have been asked if we have made meaningful progress in my time on the Board and I do, I have never been so secure in my belief that we will help patients, that we will make a difference, I know this will happen.”

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today’s most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world’s largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov
